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New Data Reinforce the Benefit of Early Preventative Treatment with Chugai's Hemlibra for Babies with Severe Hemophilia A

- Phase III HAVEN 7 primary data presented at ASH 2023 provide additional confidence in the favorable efficacy and safety profile of subcutaneous Hemlibra given soon after birth[1]
- At nearly two years median follow-up in the descriptive, single-arm study, no babies experienced spontaneous bleeds requiring treatment, and all treated bleeds were as a result of trauma[1]
- Safety results were consistent with previous studies of Hemlibra, with no new safety signals observed[1]
- The HAVEN 7 study was developed in collaboration with the hemophilia A community, to generate additional evidence for the prophylactic treatment of infants with hemophilia A

TOKYO, December 11, 2023 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that the primary analysis of the Phase III HAVEN 7 study reinforced the efficacy and safety of Hemlibra® (generic name: emicizumab) in previously untreated or minimally treated infants with severe hemophilia A without factor VIII inhibitors. Results showed that Hemlibra achieved meaningful bleed control in babies up to 12 months of age, and was well tolerated.[1] The new data were presented at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition taking place 9-12 December 2023, in San Diego, California, and included in the press program.

"Hemlibra, which can be administered subcutaneously, is an option to reduce the treatment burden for infants who have difficulty with intravenous administration in the treatment of severe hemophilia A to prevent bleeding. In this study, Hemlibra demonstrated effective bleeding control in infants for the first time. This complements data across a wide range of ages shown in previous clinical trials and supports earlier initiation of Hemlibra treatment aimed at preventing bleeding in infants. We remain committed to building evidence including long-term data to support the safe use of this drug for those who need it," said Dr. Osamu Okuda, Chugai's President and CEO.

The burden of severe hemophilia A in babies and on their parents and caregivers is significant. The World Federation of Hemophilia treatment guidelines consider the standard of care in hemophilia to be regular prophylaxis initiated at a young age, as studies have shown this improves long-term outcomes, while reducing the risk of intracranial hemorrhage.[2-4] However, for many babies with hemophilia A, prophylaxis is not started until after the first year of life.[5-8] Hemlibra, which is already approved and being used to treat babies with hemophilia A, provides a flexible treatment option that can be administered subcutaneously from birth at different dosing frequencies for maintenance dosing.[9]

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The HAVEN 7 study is a Phase III, descriptive, single-arm study, set up in collaboration with the hemophilia A community to evaluate the efficacy, safety, pharmacokinetics and pharmacodynamics of subcutaneous Hemlibra in infants with severe hemophilia A without factor VIII inhibitors. These results, which included data from 55 participants, showed that at 101.9 weeks median follow-up, 54.5% of participants (n=30) did not have any bleeds that required treatment, while 16.4% (n=9) did not have any treated or untreated bleeds at all. There were no spontaneous bleeds requiring treatment in any participant, and all treated bleeds were as a result of trauma. A total of 207 bleeds occurred in 46 participants (83.6%); 87.9% of these were as a result of trauma. Model-based annualized bleeding rate (95% CI) was 0.4 (0.30-0.63) for treated bleeds. No new safety signals were observed and there were no treatment-related serious adverse events, intracranial hemorrhages or deaths reported. 3.6% of participants (n=2) tested positive for factor VIII inhibitors which may be a consequence of reduced factor VIII usage in participants treated with Hemlibra, and no participant tested positive for anti-drug antibodies.[1] Results were consistent with positive results from the interim analysis and from previous Phase III HAVEN studies.[10-14]

The results of additional research on biomarkers in the HAVEN 7 study were also presented at ASH, and were supportive of the study's primary efficacy analysis. This additional research showed that the pharmacodynamic profiles of Hemlibra in babies were consistent with those previously observed in older children and adults with hemophilia A. The data showed that Hemlibra exhibits the expected pharmacodynamic response, despite the reduced presence of the clotting factors that Hemlibra binds to in this age group.[15]

The HAVEN 7 study results complement data from the broader, pivotal HAVEN clinical program, providing insights into the evolution of hemophilia A in babies, and the impact of initiating preventative treatment from birth. The primary analysis is being followed by a seven year extension period.[1]

About Hemlibra

Hemlibra is a bispecific monoclonal antibody created with Chugai's proprietary antibody engineering technologies. The drug is designed to bind factor IXa and factor X. In doing so, Hemlibra provides the cofactor function of factor VIII in people with hemophilia A, who either lack or have impaired coagulation function of factor VIII.[16,17] The product was approved by the U.S. Food and Drug Administration (FDA) in November 2017, for the first time in the world, for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients with hemophilia A (congenital factor VIII deficiency) with factor VIII inhibitors. Hemlibra has been approved in more than 115 countries for congenital hemophilia A with and without factor VIII inhibitors. In Japan, it was first approved in March 2018 for congenital hemophilia A with factor VIII inhibitors, and its indication was later expanded to include congenital hemophilia A without factor VIII inhibitors, and acquired hemophilia A.

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